A Phase I Open Label Study to Evaluate the Safety and Tolerability of ISP-001 in Patients with Mucopolysaccharidosis Type 1

Grant Award Details

A Phase I Open Label Study to Evaluate the Safety and Tolerability of ISP-001 in Patients with Mucopolysaccharidosis Type 1

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-14416

Investigator:

Name:	Robert Hayes
Institution:	Immusoft Corporation
Type:	PI

Award Value: $8,000,000

Status: Pre-Active

Grant Application Details

Application Title: A Phase I Open Label Study to Evaluate the Safety and Tolerability of ISP-001 in Patients with Mucopolysaccharidosis Type 1

Public Abstract:

Therapeutic Candidate or Device

B cells will be isolated from patients suffering MPSI. These will be transformed with a normal copy of the gene and re-introduced into the patient

Indication

Mucopolysaccharidosis I (MPSI) is a rare disease that affects predominantly children. Untreated, these patients typically die by the age of 10.

Therapeutic Mechanism

The therapeutic is a unique cell therapy. The patient's own B cells are genetically engineered to express the therapeutic enzyme IDUA which prevents the lethal accumulation of glycosaminoglycans in various tissues. The cells (know as plasma cells PCs) are then delivered back into the patient where they secrete IDUA at a therapeutic level. The long lived nature of PCs provides the patient with sustained (years) delivery of IDUA. Moreover, repeated dosing is possible, unlike with viral vectors.

Unmet Medical Need

The approach of turning the patient's B cells into drug-producing B cells offers the promise of continuous and sustained delivery of therapeutic levels of IDUA. The resulting drug levels mimic normal physiological conditions, and to penetrate tissues that do not receive sufficient levels of IDUA.

Project Objective

Phase I trial completed

Major Proposed Activities

- First in human dosing of adult patients using ISP001 material (IDUA transformed B cells) produced at the Fred Hutch
- Complete a long term (6 month) pharmacology study in mice to determine safety and efficacy of ISP001, enabling dosing of pediatric patients
- Transfer production of ISP001 to a third party commercial manufacturer, and prepare material for larger pediatric study

Statement of Benefit to California:

MPSI is a rare genetic disease with no race or ethnic predilections. Current treatment is expensive ($250K-500K/year/patient) and does not affect key manifestations of the disease. Our approach promises an economical, effective therapy. Immusoft of California has a strong relationship with an MPSI key opinion leader at UCLA. We are currently considering using clinical site(s) within the UC system for our pediatric study because they offer a broad reach to diverse communities throughout CA.
